200 new therapies and means to diagnose most rare diseases by the year 2020

Co-operation at international level to stimulate, better coordinate & maximise output of rare disease research efforts around the world
34 committed members

Europe
- E-RARE Consortium (EU)
- European Commission (EU)
- EURORDIS (EU)
- French Association against Myopathies (FR)
- French National Research Agency (FR)
- German Federal Ministry of Education and research (DE)
- Italian Higher Institute of Health Research (IT)
- Italian Telethon Foundation (IT)
- Lysogene (FR)
- Netherlands Organisation for Health Research and Development
- Prosensa (NL)
- Spanish Carlos III Health Institute (ES)
- UK National Institute for Health Research (UK)

North America
- Canadian Institutes for Health Research (CA)
- FDA Orphan Products Grants Program (US)
- Genome Canada (CA)
- Genetic Alliance (US)
- Mendelian Disorders Genome Centres (US)
- National Centre for Translational Sciences (US)
- National Cancer Institute (US)
- National Institute of Neurological Disorders and Stroke (US)
- National Institute of Arthritis and Musculoskeletal and Skin Diseases (US)
- National Institute of Child Health and Human Development
- National Eye Institute (US)
- NKT Therapeutics (US)
- NORD (US)
- Office of Rare Diseases (US)
- PTC Therapeutics (US)
- Sanford Research (US)
- Shire (US)

Asia
- BGI (CN)
- Korea National Institute of Health (KR)

Australia
- National Health and Medical Research Council
- Western Australian Department of Health
International cooperation: Working on the big challenges

- International K.O. Mouse Consortium
- International Cancer Genomics Consortium
- International Human Epigenome Consortium
- NEW: Global Research Collaboration for Infectious Disease Preparedness
- NEW: Global Alliance for Chronic Diseases
- International Rare Disease Research Consortium
- International Initiative for Traumatic Brain Injury Research
- International Human Microbiome Consortium
'Programme-level co-operation': how does it work?

- Identify common strategic goals
- Agree to share tasks (and costs)
- Issuing of calls in the cooperating country(ies)
- Independent evaluation, selection and funding of projects
- Funded projects work closely together
- Results, data and standards are being shared
R&I in Horizon 2020 (2014-2020)

DISCLAIMER: Pending formal adoption by the EU of the Horizon 2020 legal texts, the content of these slides is not definitive and so should not be taken as such, nor used or referred to as such in any way or for any purpose.
The European Union: 500 million people – 28 countries
Concept

all EU countries pulling resources into one R&I programme
Europe 2020 – Europe's growth strategy

- **Smart growth**
  Better education, more research, greater use of communication technologies

- **Sustainable growth**
  A resource-efficient, greener and more competitive economy

- **Inclusive growth**
  More and better jobs, investment in skills and training, modernisation of the labour market and welfare systems, spreading the benefits of growth to all parts of the EU

- **Good economic governance**
  Better coordination of economic policy
Europe 2020 - 5 EU headline targets

- 75% employment rate (% of population aged 20-64 years)
- 3% investment in R&D (% of EU’s GDP)
- “20/20/20” climate/energy targets
- School drop-out rates lower than 10% and at least 40% of the population aged 30-34 having completed tertiary education;
- Reducing those at risk of poverty or exclusion by at least 20 million
Investment in R&D is part of the solution to exit from the economic crises
30 years of EU funded R&I

Annual budget (in b€)

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<td>3,3</td>
<td>3,7</td>
<td>4,4</td>
<td>7,2</td>
<td>10</td>
</tr>
</tbody>
</table>
Horizon 2020

Europe 2020 priorities

International cooperation

Shared objectives and principles
Common rules, toolkit of funding schemes

Societal Challenges
- Health, demographic change and wellbeing
- Food security, sustainable agriculture, marine and maritime research, and the bio-based economy
- Secure, clean and efficient energy
- Smart, green and integrated transport
- Climate action, resource efficiency and raw materials
- Inclusive, innovative and secure societies

Industrial Leadership
- Leadership in enabling and industrial technologies (ICT, nano, materials, bio, manufacturing, space)
- Access to risk finance
- Innovation in SMEs

Excellent Science
- European Research Council
- Future and Emerging Technologies
- Marie Skłodowska-Curie actions on skills, training and career development
- Research infrastructures

Supporting the objectives:
European Institute for Innovation and Technology
Joint Research Centre
Three priorities:

1. Excellent science
2. Industrial leadership
3. Societal challenges
Priority 1. Excellent science

What:

• World class science is the foundation of tomorrow’s technologies, jobs and wellbeing

• Europe needs to develop, attract and retain research talent

• Researchers need access to the best infrastructures
Priority 1: Excellent science
What:

**European Research Council**
Frontier research by the best individual teams

**Future and Emerging Technologies**
Collaborative research to open new fields of innovation

**Marie Curie actions**
Opportunities for training and career development

**Research infrastructures** (including e-infrastructure)
Ensuring access to world-class facilities
Priority 2. Industrial leadership

Why:

- **Strategic investments in key technologies** (e.g. advanced manufacturing, micro-electronics) underpin innovation across existing and emerging sectors
- Europe needs to attract more private investment in research and innovation
- Europe needs more innovative SMEs to create growth and jobs
**Priority 2 Industrial Leadership**

**What:**

<table>
<thead>
<tr>
<th>Leadership in enabling and industrial technologies (ICT, nanotechnologies, materials, biotechnology, manufacturing, space)</th>
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<tbody>
<tr>
<td><strong>Access to risk finance</strong></td>
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<tr>
<td>Leveraging private finance and venture capital for research and innovation</td>
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<tr>
<td><strong>Innovation in SMEs</strong></td>
</tr>
<tr>
<td>Fostering all forms of innovation in all types of SMEs</td>
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</tbody>
</table>
Priority 3. Societal challenges

Why:

- Concerns of citizens and society/EU policy objectives (climate, environment, energy, transport etc) cannot be achieved without innovation

- Breakthrough solutions come from multi-disciplinary collaborations, including social sciences & humanities

- Promising solutions need to be tested, demonstrated and scaled up
Priority 3 Societal challenges
What:

<table>
<thead>
<tr>
<th>Health, demographic change and wellbeing</th>
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<tbody>
<tr>
<td>Food security, sustainable agriculture, marine and maritime research &amp; the bioeconomy</td>
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<tr>
<td>Secure, clean and efficient energy*</td>
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<tr>
<td>Smart, green and integrated transport</td>
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<tr>
<td>Climate action, resource efficiency and raw materials</td>
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<tr>
<td>Inclusive, innovative and secure societies</td>
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</tbody>
</table>
Horizon 2020
How?

• Collaborative research projects require at least three legal entities from different EU Member States

• Activities requiring at least one legal entity
  – European Research Council
  – Training & mobility action
  – SME instrument
  – Coordination & support actions

• Calls for proposals open to organizations from countries outside Member States and Associated Countries
Horizon 2020 Partnerships

Innovation Investment Package

10 Public-public partnerships and Public-private partnerships presented -10 July 2013- proposed EU contribution 22 Billion Euro

Innovative Medicines Initiative 2, Clean Sky 2, Fuel Cells and Hydrogen 2, Bio-based Industries, Electronics (ECSEL), SESAR (European Air Traffic Management System), European and Developing Countries Clinical Trials Partnership (EDCTP2), The European Metrology Programme for Research and Innovation (EMPIR), Eurostars 2 (to stimulate growth and job creation by supporting innovative SMEs), Active and Assisted Living Research and Development Programme
Marie Skłodowska-Curie Actions (MSCA)

Marie Curie Actions - Research Fellowship Programme
Marie Curie Fellowships are European research grants available to researchers regardless of their nationality or field of research. In addition to generous research funding scientists have the possibility to gain experience abroad and in the private sector, and to complete their training with competences or disciplines useful for their careers. Read more about various funding schemes and requirements for applying.

www.ec.europa.eu/research/mariecurieactions
## Training Actions

<table>
<thead>
<tr>
<th>Innovative Training Networks</th>
<th>ITN</th>
<th>Support for doctoral and early-stage training Training Networks, European Industrial Doctorates, European Joint Doctorates</th>
</tr>
</thead>
<tbody>
<tr>
<td>Individual Fellowships</td>
<td>IF</td>
<td>Support for experienced researchers undertaking international and inter-sector mobility: European Fellowships and Global Fellowships Dedicated support for career restart and reintegration</td>
</tr>
<tr>
<td>Research and Innovation Staff Exchange</td>
<td>RISE</td>
<td>International and inter-sector cooperation through the exchange of staff</td>
</tr>
<tr>
<td>Co-funding of programmes</td>
<td>COFUND</td>
<td>Co-funding of regional, national and international programmes: - doctoral programmes - fellowship programmes</td>
</tr>
</tbody>
</table>
What is ERC?

The ERC supports excellence in frontier research through a bottom-up, individual-based, pan-European competition.
## ERC - 25 panels for all areas of science

<table>
<thead>
<tr>
<th>Physical Sciences &amp; Engineering</th>
<th>Mathematics</th>
<th>Fundamental constituents of matter</th>
<th>Condensed matter physics</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Physical and analytical chemical sciences</td>
<td>Synthetic chemistry and materials</td>
<td>Computer science and information technology</td>
</tr>
<tr>
<td></td>
<td>Synthetic chemistry and materials</td>
<td>Artificial intelligence</td>
<td>Systems and communication engineering</td>
</tr>
<tr>
<td></td>
<td>Synthetic chemistry and materials</td>
<td>Computer science and information technology</td>
<td>Products and processes engineering</td>
</tr>
<tr>
<td></td>
<td>Synthetic chemistry and materials</td>
<td>Computer science and information technology</td>
<td>Universe sciences</td>
</tr>
<tr>
<td></td>
<td>Synthetic chemistry and materials</td>
<td>Computer science and information technology</td>
<td>Earth system science</td>
</tr>
<tr>
<td>Social Sciences &amp; Humanities</td>
<td>Economics</td>
<td>Institutions, values, beliefs and attitudes</td>
<td>Environment, space and population</td>
</tr>
<tr>
<td></td>
<td>Sociology</td>
<td>Individuals, institutions and markets</td>
<td>The Human Mind</td>
</tr>
<tr>
<td></td>
<td>Political science</td>
<td>Institutions, values, beliefs and attitudes</td>
<td>Cultures and cultural production</td>
</tr>
<tr>
<td></td>
<td>Cultural policy</td>
<td>Institutions, values, beliefs and attitudes</td>
<td>The study of the human past</td>
</tr>
<tr>
<td>Life Sciences</td>
<td>Molecular and structural biology and biochemistry</td>
<td>Genetics, genomics, bioinformatics and systems biology</td>
<td>Cellular and developmental biology</td>
</tr>
<tr>
<td></td>
<td>Physiology, pathophysiology and endocrinology</td>
<td>Neurosciences and neural disorders</td>
<td>Immunity and infection</td>
</tr>
<tr>
<td></td>
<td>Evolutionary, population and environmental biology</td>
<td>Diagnostic tools, therapies and public health</td>
<td>Applied life sciences and biotechnology</td>
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</tbody>
</table>
EU-funded Health Research

Iiro Eerola
Scientific Project Officer
Unit for Personalised Medicine
Directorate for Health Research
DG Research & Innovation
European Commission
The FP7 Health Programme

The largest multi-national fund

• for collaborative research, global consortia, and public-private co-operation

• to fund excellent applied health research and innovation

• to bring together leading players from Europe and across the globe

• to tackle key European and global health challenges
State of play

Key figures

6 b€ invested to date
1,000 projects
11,000 teams
3,500 organisations
130 countries

First outcomes
(on 281 closed projects)

200 patent applications
9,000 publications
3,3 average SJR* publication
30 spin-offs created

* SCImago Journal Ranking
EU health research delivers results for patients

• Treat OA - better treatment & diagnosis of osteoarthritis

• EU contribution: €11.9 million

• Helped develop TIGENIX' lead product ChondroCelect© for cartilage regeneration in the knee

• This product is the first ATMP (Advanced Therapy Medicinal Product) approved in the EU.

www.treatoa.eu
EU health research concentrates scarce resources

- Alpha Man project is developing a promising treatment for patients suffering from the rare disease, alpha-mannosidosis.

- Alpha mannosidosis has a prevalence of 1 in 500,000 people.

- A biotechnologically derived human enzyme has successfully completed a phase 2a clinical trial.

- Three projects from 2001 to 2013 have led to this stage, with a total EU contribution of ~€10 million.

- European research has been crucial given the low case numbers per member state and the limited incentive for private investment.
EU level health research co-ordinates

- ...objectives which can only be achieved at European / global level
- e.g. the Blueprint project – Europe's co-ordinated contribution to the International Human Epigenome Consortium
- Agreed goal of IHEC: generate 1000 epigenomes
- SMEs are embedded in the consortium, paving the way for the next phase of research, drug development.

**Duration:** October 2011 – April 2016  
**Budget:** EUR 39.9 million (EU contribution EUR 30 million)

**Partners/countries**  
BLUEPRINT involves 41 partners from nine EU Member States, Switzerland and Israel. A full list is available at www.blueprint-epigenome.eu

**Project website**  
www.blueprint-epigenome.eu
IMI: Europe's highway to better medicines

- New type of public/private partnership
- 1:1 funding, joint decision making
- All EU funding goes to SMEs, academia, patient organisations and regulatory agencies
- Large pharmaceutical industry, represented by EFPIA, contributes in-kind

www.imi-europa.eu
Europe's highway to better medicines
(data from 2012 – currently 40 projects)

- 508 Academic & research teams
- 341 EFPIA teams
- 92 SMEs
- 17 patient org
- 10 regulators
- 37 projects
- 603 million EUR IMI JU cash contribution
- 600 million EUR EFPIA in kind contribution
- ~ 3500 researchers
- > 200 publications

Project example:
Europe’s highway to better medicines
Be The Cure for Rheumatoid Arthritis

- 24 academic partners, 1 SME, 9 EFPIA partners
- 5 years, €38 million
- Started in April 2011
In the pipeline: IMI2

• Commission proposal published in July

• Mission: create an even more powerful research and innovation machine – with broader objectives and a wider range of activities and partners

• Key objectives:
  • pave the way for the breakthrough vaccines, medicines and treatments which we will need in the near future
  • Enable top quality research and innovations with great public health benefits and commercial possibilities
European & Developing Countries Clinical Trials Partnership (EDCTP)

- A first in 'Joint Programming' between European national research programmes
- Test-bed to pioneer the coordination, joint implementation and integration of European national research programmes

www.edctp.org
EDCTP-funded clinical trials 2003-2011

**HIV/AIDS**
- 27 clinical trials
- Drugs: 14
- Vaccines: 9
- Microbicides: 4

**Tuberculosis**
- 18 clinical trials
- Drugs: 7
- Vaccines: 7
- Diagnostics: 4

**Malaria**
- 12 clinical trials
- Drugs: 10
- Vaccines: 2
In the pipeline: EDCTP2

- **EDCTP2 launch in early 2014 expected with:**
  
a) Larger scope (including neglected infectious diseases, all clinical phases, diagnostics and delivery optimisation)
  
b) Bigger budget
  
c) Longer duration

- **16 European countries have committed to spend almost €600 million on EDCTP2**

- **EU will provide matching co-funding through its Horizon 2020 programme**
US organisations in FP7 Health

- **152 US participations:**
  - 63 Universities
  - 39 Research organisations
  - 27 SMEs
  - 13 Foundations
  - 10 Other (International organisations, FDA etc.)

- **Receiving 51 million €**
5 main US participating organisations

- University of California: 10
- The Brigham and Women's Hospital: 4
- University of Pennsylvania: 4
- Duke University: 3
- Mount Sinai School of Medicine: 3
BESTCILIA: Better Experimental Screening and Treatment for PCD

- Primary Ciliary Dyskinesia (PCD) is a rare, genetically heterogeneous disorder causing severe, chronic destructive airway disease with progressive loss of lung function
- The main goal of the project is to characterise the clinical course and improve the diagnosis and treatment of PCD patients

Participants: DE, CH, CY, DK, EL, NL, PL, UK, US (University of Miami)

EU contribution: € 2.9 million

www.bestcilia.eu
IDEA: Dissecting the Immunological Interplay between Poverty Related Diseases and Helminth infections: An African-European Research Initiative

- The primary objective of IDEA is to determine whether and how the presence of worm infections modulate immune responses specific to HIV, TB and Malaria, the clinical course of these diseases, and vaccination and vaccine-induced immune responses

Participants: 6 EU countries, 5 African countries and the US (Vaccine & Gene Therapy Institute Florida)

EU contribution € 10.3 million

www.idearesearch.eu
Health R&I in Horizon 2020 (2014-2020)
Health Challenge

Understanding health, wellbeing and disease

Preventing disease

Treating and managing disease

Active ageing and self-management of health

Methods and data

Health care provision and integrated care

Unravelling the complexity of health and disease

Towards personalised, safe and more effective health interventions

Advancing active and healthy ageing

Improving health information, data exploitation and providing an evidence base for health policies and regulation

Integrated, sustainable, citizen centred care
How to participate in Horizon 2020
The basics (based on health programme)

• **1b€/year** devoted to health research in Horizon 2020

• **One rule**: Minimum 3 partners from 3 EU countries + anyone else from anywhere in the world

• **Project duration**: 3-5 years

• **Call publication**: planned December 2013
Why should I go for EU funding?

1. Build a strong network with top EU organisations and scientists
2. Share complementary knowledge & experience
3. Receive funding for world-class research
4. Get a first-hand experience with EU businesses and healthcare markets
5. Discover Europe: culture, art, food and wines ☺!
How to apply?

1. Read the call, identify a relevant topic
2. Find EU partners
3. Write and submit your proposal
4. Get funded (~10% success rate)!

http://ec.europa.eu/research/participants/portal
Find a partner for your project

- Get in touch with your national contact point: http://ec.europa.eu/research/participants/portal/page/nationalcontactpoint
- Fit for Health: http://www.fitforhealth.eu
- Health competence: http://www.healthcompetence.eu
- IMI partnering platform: http://www.imi-partnering.eu
We need your expertise

• Register as an expert
• ... or make sure that your profile is up to date

http://ec.europa.eu/research/participants/portal/page/welcomeexpert
Thank you!

Irene.Norstedt@ec.europa.eu
Iiro.Eerola@ec.europa.eu

http://ec.europa.eu/research/health